

Case Study: strategic support with new rare disease indication



GRG supporting HEOR strategy for expanded access for a rare disease treatment

- Novel treatment applies to several indications associated with spectrum of rare genetic metabolic diseases.
- Some indications have received FDA and EMA approval, but more indications in pipeline.



Challenge:

- Client requested support in planning short- and long-term HEOR strategies.
- Since indications were rare diseases with limited existing data on the population, developing an evidence base required a unique approach, including planning natural history studies, registry-based analyses, and *de novo* data collection.



Approach

GRG collaborated with the client to develop a customized HEOR strategy to build an evidence base for pipeline indications.

- GRG also developed a custom integrated evidence platform for internal communication and knowledge management across the global organization.



GRG Solutions

- ✓ Value communication and HEOR strategy
- ✓ Dissemination of findings through presentations and publications
- ✓ Analysis of clinical trial data, including patient reported outcomes
- ✓ Evidence synthesis and literature reviews



Dedicated team composition

